

531,349

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau



(43) International Publication Date
29 April 2004 (29.04.2004)

PCT

(10) International Publication Number
WO 2004/035765 A2

- (51) International Patent Classification⁷: C12N
Shepard Drive, Lansdale, PA 19446 (US). MCCALLUS, Daniel, Edward [US/US]; 312 Gumbes Road, Oaks, PA 19456 (US).
- (21) International Application Number: PCT/US2003/033466
(74) Agent: BIEKER-BRADY, Kristina; Clark & Elbing LLP, 101 Federal Street, Boston, MA 02110 (US).
- (22) International Filing Date: 20 October 2003 (20.10.2003)
- (25) Filing Language: English
(81) Designated States (*national*): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CH, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NI, NO, NZ, OM, PG, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, SY, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.
- (26) Publication Language: English
- (30) Priority Data:
60/419,532 18 October 2002 (18.10.2002) US
60/421,757 28 October 2002 (28.10.2002) US
- (63) Related by continuation (CON) or continuation-in-part (CIP) to earlier applications:
US 60/419,532 (CIP)
Filed on 18 October 2002 (18.10.2002)
US 60/421,757 (CIP)
Filed on 28 October 2002 (28.10.2002)
- (71) Applicant (*for all designated States except US*): NUCLEONICS, INC. [US/US]; 14 Spring Mill Drive, Malvern, PA 19355 (US).
- (72) Inventors; and
(75) Inventors/Applicants (*for US only*): PACHUK, Catherine, J. [US/US]; 3212 New Park Circle, Lansdale, PA 19446 (US). SATISHCHANDRAN, C. [US/US]; 605
- (84) Designated States (*regional*): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).
- Published:**
— without international search report and to be republished upon receipt of that report
- For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.*

(54) Title: DOUBLE-STRANDED RNA STRUCTURES AND CONSTRUCTS, AND METHODS FOR GENERATING AND USING THE SAME

Abstract: The present invention relates to novel double-stranded RNA (dsRNA) structures and dsRNA expression constructs, generating them, and methods of utilizing them for silencing genes. Desirably, these methods specifically inhibit the expression of one or more target genes in a cell or animal (e.g., a mammal such as a human) without inducing toxicity. These methods are used to prevent or treat a disease or infection by silencing a gene associated with the disease or infection. The invention also includes methods for identifying nucleic acid sequences that modulate a detectable phenotype, such as the function of a cell, the expression of a gene, or the biological activity of a target polypeptide.

BEST AVAILABLE COPY